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## Evaluation of Safety and Preliminary Efficacy of Escalating Doses of GRNOPC1 in Subacute Spinal Cord Injury

### Grant Award Details

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Evaluation of Safety and Preliminary Efficacy of Escalating Doses of GRNOPC1 in Subacute Spinal Cord Injury

**Grant Type:** Targeted Clinical Development

**Grant Number:** CT1-05168

**Investigator:**

<b>Name:</b>	Jane Lebkowski
<b>Institution:</b>	Geron Corporation
<b>Type:</b>	PI

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**Disease Focus:** Neurological Disorders, Spinal Cord Injury

**Human Stem Cell Use:** Embryonic Stem Cell

**Award Value:** \$6,405,771

**Status:** Closed

### Grant Application Details

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**Application Title:** Evaluation of Safety and Preliminary Efficacy of Escalating Doses of GRNOPC1 in Subacute Spinal Cord Injury

**Public Abstract:**

The proposed project is designed to assess the safety and preliminary activity of escalating doses of human embryonic stem cell (hESC) derived oligodendrocyte progenitor cells for treatment of spinal cord injury. Oligodendrocyte progenitor cells have two important functions: they produce neurotrophic factors which stimulate the survival and growth of neurons (nerve cells) after injury, and they mature in the spinal cord to produce myelin, the insulation which envelops neuronal axons (nerve cell bodies responsible for conduction) and facilitates unimpeded nerve impulse conduction. After extensive efficacy and safety testing, clinical testing of this product was initiated in 2010.

Clinical testing is being initiated in paraplegic patients with neurologically complete thoracic injuries (i.e., those in which no motor or sensory function remains below the level of the injury). In the first cohort, a dose equivalent to the lowest efficacious dose observed in preclinical rodent studies is being administered. During the course of the proposed program, clinical safety studies testing increasing doses will be conducted. Upon demonstration of safety, clinical testing will be expanded to tetraplegic patients (complete cervical injuries) and to patients with incomplete thoracic injuries for additional safety testing. In each of the proposed studies, preliminary evidence of activity will be monitored using measures of improved neurological function and performance of daily living activities.

The project plan also includes the manufacture of cells to be used in the clinical trials and additional supporting activities. By completion of the proposed project, we expect to have accumulated substantial safety data and preliminary efficacy data in three different patient subpopulations. This data will provide key information to inform the design and execution of advanced efficacy studies.

**Statement of Benefit to California:**

The proposed project has the potential to benefit the state of California through 1) providing improved medical outcomes for patients with spinal cord injury and their families, 2) increasing California's leadership in the emerging field of stem cell research, and 3) preserving and creating high quality, high paying jobs for Californians.

Over 12,000 Americans suffer spinal cord injuries each year, and approximately 1.3 million people in the US are estimated to be living with spinal cord injuries. Although specific estimates for the state of California are not available, it is known that the majority of spinal cord injuries result from motor vehicle accidents, falls, acts of violence and recreational sporting activities, all of which are prevalent in California. Spinal cord injury affects not only the patient but family members, friends, healthcare workers and employers. It is estimated that one year after injury, only 11.6% of spinal cord injury patients are employed, and that spinal cord injuries cost \$40.5 billion annually in the US. As the most populous state, California is disproportionately affected, negatively impacting our productivity, healthcare system and public finances. There are currently no approved therapies for the treatment of spinal cord injury. The product described in this application has initiated phase 1 clinical testing in patients with complete thoracic spinal cord injury. Even partial correction of any of the debilitating consequences of spinal cord injury could potentially enhance activities of daily living and increase employment while decreasing reliance on attendant care and subsequent medical interventions.

California has a history of leadership in biotechnology, and is emerging as a leader in the development of stem cell therapeutics. Cutting edge stem cell research, in many cases funded by CIRM, is already underway in academic research laboratories and biotechnology companies throughout the state. The proposed project has the potential to further increase California's leadership in the field of stem cell therapeutics through the performance of the first clinical testing of an hESC-derived therapy.

The applicant has been located in California since its inception, and currently employs 182 full-time employees at its California headquarters with more than 50% of employees holding an advanced degree. These positions are highly skilled positions, offering competitive salaries and comprehensive benefits. The successful performance of the proposed project would enable significant additional jobs creation in preparation for pivotal trials and product registration.

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